Paediatric Clinical Trials
20th & 21st March 2013
Copthorne Tara Kensington, London, UK

KEY SPEAKERS INCLUDE:

David McIntosh
Global Scientific Affairs Senior Expert
Novartis

Lisa Moore-Ramdin
Medical Affairs Manager
GlaxoSmithKline

Luc De Schaepdrijver
Scientific Director
Johnson & Johnson

Philippe Auby
Regional Medical Director
Lundbeck

Paolo Rossi
Head of Division of Immunology and Infectious Disease, PDCO Member
Children’s Hospital Bambin Gesu

Bruno Reigner
Expert Scientist
Roche

Daniel Bar-Shalom
Associate Professor
University of Copenhagen

Florentine Nieuwmeyer
Associate Scientific Director
Astellas

Noel Cranswick
Director Clinical Pharmacology Unit
Royal Children’s Hospital Melbourne

Biljana Jovanova
Head of Department
University Clinic for Children’s Diseases
Macedonia

KEY BENEFITS OF ATTENDING:

• Strategies for patient recruitment and retention
• How to overcome obstacles in paediatric clinical trial design
• Evaluate benefits of locating clinical trials in emerging markets
• Consider new EMA policies and guidelines on Paediatric Investigation Plans (PIP)
• Discuss the PIP negotiation process with the EMEA/PDCO
• Understand a patient representative perspective
• Hear the latest in paediatric vaccine development

PLUS ONE INTERACTIVE HALF-DAY PRE-CONFERENCE WORKSHOP
Tuesday 19th March 2013, Copthorne Tara, London, UK

Managing the Paediatric Investigation Plan (PIP) in Drug Development.
From preparation to successful negotiation, execution and sometimes modification

Workshop Leader:
Klaus Rose, Managing Director, Klausrose Consulting
13.30 - 17.30

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08.30 Registration and Coffee

09.00 Chairman’s opening remarks
Klaus Rose, Managing Director, klausrose Consulting

NEW EMA POLICIES AND GUIDELINES ON PAEDIATRIC INVESTIGATION PLANS

09.10 Paediatric Legislation: Challenges and Impact on Drug Development
• Background of EU & US paediatric legislation
• Scientific & operational challenges of the Paediatric Investigation Plan (PIP)
• Better medicines for children Vs medicines for adults developed in children
• A tentative assessment of the impact of the EU legislation
Klaus Rose, Managing Director, klausrose Consulting

09.50 A legal perspective on the activities of the Paediatric Committee (PDCO)
• Assessing the content of PIPs
• Working with the CHMP or EU to adopt opinions on quality, safety and efficacy
• Generating advice for developments in research
Genevieve Michaux, Lawyer, Covington & Burling

10.30 Morning Coffee

11.00 Do I really need a PIP? The importance of understanding the impact of patent / SPC status
• What does the pharmaceutical and SPC legislation say?
• How do I know if my patent qualifies for an SPC?
• What is the impact of the ECJ Neurim judgement?
• Who is responsible for deciding what?
• What are the consequences of getting it wrong?
Steve Pinder, Director, Envestia

CLINICAL TRIAL DESIGN AND OVERCOMING PRACTICAL ISSUES

11.40 Problems in organizing clinical trials in low income countries
• Recruiting patients
• Accredited laboratories
• Ethical comity
• Legal issues
• Parents education and compliance
Biljana Choneska Jovanova, Head of Department, University Clinic for Children’s Diseases, Macedonia

12.20 Networking Lunch

13.40 Non-clinical safety studies in juvenile animals
• Non-clinical studies: decision strategies and place in paediatric drug development
• Juvenile animal toxicity studies (JAS): why ? when ? how ?
• Value of JAS for clinical paediatric trials and for the product label
• Case examples
Luc De Schaepdrijver, Scientific Director, Johnson & Johnson

14.20 Designing and implementing paediatric clinical trials: the example of paediatric psychopharmacology
• Understanding ethical and scientific challenges
• Overcoming practical issues
• Example of published or ongoing paediatric clinical trials
• Practical examples
Philippe Auby, Regional Medical Director, Lundbeck

ETHIC COMMITTEES AND REVIEWS

15.00 Regulatory perspective on paediatric trials
• Activities of the PDCO
• Working together to further drug development
• Methodological issues in trial design
Paolo Rossi, Head of Division of Immunology and Infectious Disease, PDCO Member, Children’s Hospital Bambin Gesu

15.40 Afternoon Tea

16.00 Focusing your paediatric IRB/ethics committee applications to help avoid study time delays
• Background and overview of key ethical issues
• An ethics committee perspective
• Learning from past experiences and case studies
• Implementing Good Ethical Practice© to improve success
Jane Lamprill, Paediatric Research Advisor & Trainer, Paediatric Research Consultancy

16.40 Patient representative perspectives
• The importance of involving patients and families in the design and delivery of research (explanation of the MCRN model)
• Examples of involvement – impact of patients and families
• Disseminating research findings to patients and families to encourage wider participation in trials
Jennifer Newman, PPI Manager, MCRN

17.20 Chairman’s Closing Remarks

17.30 End of Day One

Who should attend:
Pharmaceutical and Biotech professionals in positions such as:
• Clinical Trial Project Manager
• Director of Clinical Research
• Clinical Research Associate
• R&D Project Leader
• Head of Paediatrics
• Director of Regulatory Affairs
• Director of Medical Affairs
• Chief Medical Officer
• Toxicologist
• Clinical Pharmacologist
• Senior Medical Advisor
• Formulation Development
• Patient Safety & Risk Management
• Managing Director

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Register online at www.paediatric-trials.co.uk • Alternatively fax
08.30 Re-registration and Coffee

09.00 Chairman’s opening remarks
Lisa Moore-Ramdin, Senior Medical Advisor, Paediatric Vaccines, GlaxoSmithKline

09.10 Paediatric Vaccine Development
• Licensure bases on immunogenicity of efficacy
• Post-authorization safety commitments in immunisation
• Herd protection and the success of conjugate vaccines
• Viral vaccines eg influenza, rotavirus and human papillomavirus
• Re-emerging infections and emerging infections: trials
Reverse vaccinology
David McIntosh, Global Scientific Affairs Senior Expert, Novartis

09.50 Developments in Paediatric Blood Sampling
• EU Directives, ICH GCP, and Local Guidelines
• Minimum blood volumes
• Lab facilities
• Staff training in venepuncture and logistics of sampling
• Consent/Assent
Lisa Moore-Ramdin, Medical Affairs Manager, GlaxoSmithKline

10.30 Morning Coffee

10.50 Paediatric Medicine Management
• What makes children different? - Is it just size?
• Quality Assurance of paediatric medicines
• Ensuring patient compliance
• Best practice techniques
Steve Tomlin, Consultant Pharmacist, Evelina Children’s Hospital

11.30 Paediatric formulation development
• Designing accurate and safe formulation
• Specific needs for paediatric medicines – including those related to physiological and anatomical differences
• Developing age-appropriate formulations
Hannah Bachelor, Medicines for Children Research Network Formulation Fellow, University of Birmingham

12.10 Formulation development of an acceptable drug product for the paediatric population
• Regulatory guidance and dosage form acceptability
• Patient acceptability
• Technical feasibility of dosage form development
Florentine Nieuwmeyer, Associate Scientific Director, Astellas

12.50 Networking Lunch

14.10 Tackling the Paediatric Dosage-Form Formulation Issues
• Pairing the right dosage form with the [actual] patient
• Stability considerations
• Dose adjustment
• Choice of excipients [read: dodging trouble]
Daniel Bar-Shalom, Associate Professor, University of Copenhagen

14.50 Clinical pharmacology considerations in paediatric drug development: dosing, extrapolation and the use of modeling and simulations
• The high failure rate in paediatric confirmatory trials, exploring possible reasons
• A two-step approach to avoid dosing mistakes
• Extrapolation: current practice and recent developments
• Modeling and simulation approaches: timing and benefits
Bruno Reigner, Expert Scientist, Roche

15.00 Afternoon Tea

15.30 Multi-Centre Paediatric Trials
• Large early phase pediatric clinical trials centers
Noel Cranswick, Director, Clinical Pharmacology Unit, Department of Medicine, Royal Children’s Hospital Melbourne

15.50 Multi-Centre Clinical Trials
• Reducing barriers to international market entry
• Maintaining relationships with local health authorities
• Managing local resources directly
• Improving flexibility
Colin Powell, Senior Lecturer, Cardiff University

17.10 Chairman’s Closing Remarks

17.20 Close of Day Two

Day Two | Thursday 21st March 2013

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Supported by
Overview of workshop
The workshop will be a hands-on exercise leading through the stages of the EU Paediatric Investigation Plan (PIP) from preparation through negotiation to execution and final report. Topics will include the forms to fill in, key considerations for the internal PIP preparation within a company, the steps of PIP submission to EMA / PDCO, and the challenges of PIP execution and reporting its results.

You should attend this workshop
• If you have to manage the entire PIP (paediatric investigation plan) a procedure or parts thereof
• If you are involved in issues that touch paediatric drug development and you want to understand the logic underlying EU requirements
• If you are involved in paediatric clinical trials and you would like to know why and how paediatric clinical trials are planned

Agenda
13.00 Registration and coffee
13.30 Key elements of EU & US pediatric legislation
• Introduction & welcome
• Key elements of EU & US pediatric legislation
• EU Pediatric coordinator, rapporteur, co-rapporteur, EU Forms
• Request for a PIP vs. Request for a Waiver
14.15 Physiology in children as opposed to adults
• Absorption, Distribution, Metabolisation & Excretion in Children
• Consequences for drug therapy
• Use of pediatric ADME in the PIP
15.00 Coffee
16.15 EMEA / PDCO PIP decisions in various indications
• Going together through the EMA pediatric website
• Discuss the case samples on the decision page
• Identify key elements of EMA / PDCO pediatric decisions
17.00 Where to find help: EU and US paediatric research networks, CROs, consultancies
• European national paediatric research networks
• European disease-specific paediatric research networks
• US networks, CROs, consultancies
17.30 Close of workshop

About the workshop host
Dr. Klaus Rose is CEO of Klausrose Consulting, Switzerland, advising on pediatric drug development and how to comply with FDA & EMA pediatric requirements. He first studied Latin languages & psychology and then medicine. After postgraduate clinical training in General Medicine he joined the pharmaceutical industry 1991. He held various positions in R&D and medical affairs, was Global Head Pediatrics Novartis 2001 - 2005 and Global Head Pediatrics Roche 2005 - 2009. After a year with a regulatory consultancy he established his own business. Dr. Rose is a frequent speaker on international conferences on pediatric drug development and publishes on a regular base. The second edition of "Guide to Paediatric Drug Development and Clinical Research", co-edited with Professor van den Anker, was released 2010.

About the organisation
Klausrose Consulting, Switzerland, www.klausrose.net, supports pharmaceutical companies, CROs and other health care providers in all aspects of paediatric drug development.
### JANUARY

- **Biomarkers Summit**  
- **Social Media in the Pharma Industry**  
  23 – 24 January 2013, London
- **Quality by Design**  
  23 – 24 January 2013, London
- **Pre-Filled Syringes**  
  28 – 29 January 2013, London
- **Pharmaceutical Microbiology**  

### FEBRUARY

- **Parallel Trade**  
  6 – 7 February 2013, London
- **Advances and Progress in Drug Design**  
  18 – 19 February 2013, London
- **Lyophilisation - Freeze Drying in Pharmaceuticals and Biopharmaceuticals**  
  25 – 26 February 2013, London

### MARCH

- **Superbugs & Superdrugs - A Focus on Antibacterials**  
  4 – 5 March 2013, London
- **Imaging in Cancer Drug Development**  
  13 – 14 March 2013, London
- **Controlled Release**  
  18 – 19 March 2013, London
- **Paediatric Clinical Trials**  
  20 – 21 March 2013, London

### APRIL

- **Adaptive Designs**  
  8 – 9 April 2013, London
- **Asthma & COPD**  
  15 – 16 April 2013, London
- **Pharmaceutical Portfolio & Lifecycle Management**  
  17 – 18 April 2013, London

### MAY

- **Generics, Supergenerics & Patent Strategies**  
  13 – 14 May 2013, London
- **Pain Therapeutics**  
  20 – 21 May 2013, London
- **ADC Summit**  
  20 – 21 May 2013, London
- **Clinical Trial Logistics**  
  22 – 23 May 2013, London

### JUNE

- **RNAi & Nanotechnology**  
  5 – 6 June 2013, London
- **Biobanking**  
  24-25 June 2013, London
- **Allergies**  
  26 – 27 June 2013, London
- **ADMET**  
  26 – 27 June 2013, London

### JULY

- **Pharmacovigilance**  
  1 – 2 July 2013, London
- **Cell Culture**  
  3 – 4 July 2013, London
PAEDIATRIC CLINICAL TRIALS
Conference: Wednesday 20th & Thursday 21st March 2013, Copthorne Tara Kensington, London, UK
Workshop: Tuesday 19th March 2013, Copthorne Tara, London, UK

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